

SANFORD REPORT

Monthly Newsletter of Sanford Stem Cell Clinical Center

UC San Diego Health

Sanford Stem Cell Clinical Center

IN THIS ISSUE

First Patient Dosed in Cystinosis Clinical Trial

UC San Diego Receives \$9 Million in Grants to Pinpoint Cellular Cause of Type 1 Diabetes

What It's Like to Have Type 1 Diabetes

Working with Natural Born Killers: Using NK Cells to Improve Cancer Immunotherapies

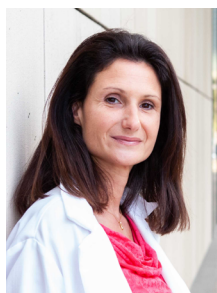
Dr. Jeremy Rich Co-Authors Glioblastoma Study Published in *Cell*

52 UC San Diego Researchers Are Most Highly Cited in Their Fields

Tribute to Bradley J. Fikes



The UC San Diego Health CIRM Alpha Stem Cell Clinic team. From left to right: Andrew Mahar, MS, Betty Cabrera, MPH, CCRC, Gina Balzon, ACRP-CP, Vicky Vardouniotis, and Julissa Gonzalez, MS. The Alpha Stem Cell Clinic helps launch and support regenerative medicine clinical trials at UC San Diego Health. A menu of services can be found [here](#).



Stephanie Cherqui, PhD, associate professor of pediatrics at UC San Diego

First Patient Dosed in Dr. Stephanie Cherqui's Phase I/II Trial of Gene Therapy for Cystinosis

The first patient has been dosed in AVROBIO's AVR-RD-04 investigational gene therapy program for cystinosis, a devastating lysosomal storage disease, in an ongoing Phase 1/2 clinical trial sponsored by academic collaborators at the University of California San Diego. The gene therapy is derived from the patient's own hematopoietic stem cells, which are genetically modified to produce functional cystinosis, a crucial protein that patients with cystinosis lack.

The trial will enroll up to six patients with cystinosis, a rare inherited disease caused by a defect in the gene that encodes for cystinosis. The cystinosis protein enables transport of the amino acid cystine out of lysosomes. When it is absent, cystine accumulates and crystalizes, causing progressive damage to the kidneys, liver, muscles, eyes and other organs and tissues. Cystinosis affects both children and adults; they face shortened life spans and often painful symptoms, including muscle wasting, difficulty breathing, blindness and kidney failure.

[Read more](#)



Maïke Sander, MD, professor of pediatrics and cellular and molecular medicine at UC San Diego

UC San Diego Receives \$9 Million in Grants to Pinpoint Cellular Cause of Type 1 Diabetes

UC San Diego School of Medicine researchers have been awarded nearly \$9 million to fund two multi-institutional research projects that use human pluripotent stem cells, CRISPR and human organoids to dissect beta cell defects and create a human cell model of type 1 diabetes aimed at identifying the elusive cellular actions leading to disease onset.

[Read more](#)



Jeremy Pettus, MD, assistant professor of medicine and principal investigator in the UC San Diego Health CIRM Alpha Stem Cell Clinic

What It's Like to Have Type 1 Diabetes

Diagnosed with type 1 diabetes at age 15, Jeremy Pettus, MD, has dedicated his career to treating others with the disease. Dr. Pettus opened up about his experience with diabetes as a teenager, research advances and how it affects his parenting. Check out the video [here](#).



Dan Kaufman, MD, PhD, professor of medicine, director of cell therapy at UC San Diego and scientific/medical director of the Advanced Cell Therapy Laboratory

Working with Natural Born Killers: Using Natural Killer Cells to Improve Cancer Immunotherapies

Natural Killer - or NK - cells are one of our immune systems most potent defenses, able to attack viral infections and destroy cells that exhibit tumorigenic characteristics.

UC San Diego physician Dr. Dan Kaufman, who has specialized in treating blood cancers for over 20 years now leads a research group at the UC San Diego Stem Cell program that is using induced pluripotent stem cells to generate NK cells that are targeted to destroy cancer cells. With the goal of providing safer, more effective, off-the-shelf cancer immunotherapies, the methods his lab has developed are being employed in a first-of-its-kind clinical trial anywhere in the world being conducted at the UC San Diego Health. Check out the UC-TV video [here](#).



Tribute to Bradley J. Fikes

Bradley J. Fikes, San Diego Union-Tribune biotech writer, died November 20, 2019 at his home in Grantville. He was 61 years old.

He was a highly regarded and beloved journalist who interpreted the science and highlighted the needs and interests of patients.

"Bradley championed their cause by telling their stories clearly," said Dr. Catriona Jamieson. "He was a serious advocate for patients. He persevered and got difficult stories right. I've always been a big fan of Bradley."

Bradley will be truly missed. [Read more](#)

Dr. Jeremy Rich Co-Authors Glioblastoma Study Published in *Cell*

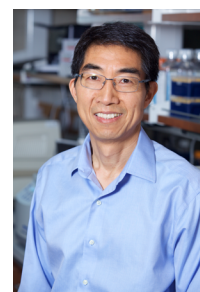
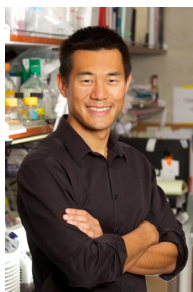
By Heather Buschman, PhD and William Lubinger, Case Western Reserve University

Extra DNA scooped up and copied alongside cancer-causing genes helps keep tumors going — elements that could represent new drug targets for brain tumors and other cancers notoriously difficult to treat

One of the ways a cancer-causing gene works up enough power to turn a normal cell into a cancer cell is by copying itself over and over, like a Xerox machine. Scientists have long noticed that when cancer-causing genes do that, they also scoop up some extra DNA into their copies. But it has remained unclear whether the additional DNA helps drive cancer or is just along for the ride.

Using human glioblastoma brain tumor samples, researchers at University of California San Diego School of Medicine and Case Western Reserve University School of Medicine have now determined that all of that extra DNA is critical for maintaining a cancer-causing gene's activation, and ultimately supporting a cancer cell's ability to survive. Comparing those findings to a public database of patient tumor genetics, they also discovered that even if two different tumor types are driven by the same cancer-causing gene, the extra DNA may differ.

The study, published November 21, 2019 in [Cell](#), could explain why drugs will often work for some cancer types but not others. [Read more](#)



From Left to Right: Gene Yeo, PhD, professor of cellular and molecular medicine, Jeremy Rich, MD, MHS, MBA, professor of medicine, and Kun-Liang Guan, PhD, Distinguished Professor of pharmacology and Moores Cancer Center. All three researchers have been impacted by Sanford Center funds.

52 UC San Diego Researchers Are Most Highly Cited in Their Fields

Fifty-two faculty members and researchers at UC San Diego are among the world's most influential in their fields. The Web of Science Group, an information and technology provider for the global scientific research community, compiled its 2019 Highly Cited Researchers list of more than 6,000 scientists from around the world whose studies were among the top 1% of most-cited publications in their field over the past 11 years.

Of these 52 researchers, three of them have received funding from the Sanford Stem Cell Clinical Center to continue their research. Geo Yeo, PhD, Jeremy Rich, MD, MHS, MBA, and Kun-Liang Guan, PhD have research labs at the Sanford Consortium for Regenerative Medicine. [Read more](#)